The **Patients as Partners** team is proud to celebrate **Women’s History Month** by highlighting three leaders and advocates for patient engagement and empowerment.

**Joanne Waldstreicher, MD**  
CMO, Johnson & Johnson

**Jamie Troil Goldfarb**  
Patient Advocate

**Lesley Gosden**  
Patient Advocate
Patients as Partners Celebrates

Joanne Waldstreicher, MD
Chief Medical Officer, Johnson & Johnson

Talks About the Office of the Chief Medical Officer and Incorporating Patient Perspectives

What sparked your interest in healthcare?

My passion for science and medicine was instilled in me at a young age by my father. He was a baker who worked harder than anyone I knew – and he never lost his desire to continue learning and growing. He loved science.

Even though he never went to college, he would read my college chemistry textbooks and discuss them with me. He was very scientific in his approach to baking, calculating ratios and percentages in his head, correcting formulas (he never called them recipes) for atmospheric pressure, etc.

My father passed this love of science on to my siblings and me, always encouraging us to strive to make the world a better place through science. The example he set led all three of us to pursue careers in medicine.

OCMO is composed of medical and scientific professionals, including safety experts, from all three sectors of Johnson & Johnson: pharmaceuticals, medical devices and consumer products. We focus on the safety of our products through people-driven processes, initiatives and policies. Every product within our company has a team of medical and scientific professionals, independent of commercial interests, responsible for evaluating safety. Our goal is to advance evidence- and science-based decision-making that is driven by bioethical principles and values.

What inspires you to keep doing the work that you do?

What inspires me every day is the potential to improve the lives of people all over the world and to know that, through science and medicine, we are truly making a difference. There are two parts to it – I call it the ‘what’ and the ‘how.’ ‘What’ we develop, the innovative products – whether in oncology, vaccines, or other areas – that make a real difference. And ‘how’ we act, how we make decisions in ways that keep patients at the center of our actions, is a relentless focus that continues to drive me.

How did you end up at Johnson & Johnson and create the Office of Chief Medical Officer?

My decision to leave academic medicine at Harvard and make a career change to industry was inspired by the realization that a lot of the really impactful research, the studies that could really make a difference for a lot of patients, comes from industry. I realized that in working with industry, I would have the opportunity to help millions of people around the world.

I started my career in various roles and therapeutic areas at Merck before joining the Janssen Pharmaceutical Companies of Johnson & Johnson nearly 20 years ago. I absolutely loved working in development – bringing new and important products to patients. I also came to understand the importance of independent input to critical decision-making. That’s why I brought the idea of having an internal but independent patient-centered organization to our CEO, which would focus primarily on safety and other important decision-making. That organization is now called The Office of the Chief Medical Officer (OCMO), which I lead.

What inspires you to keep doing the work that you do?

What inspires me every day is the potential to improve the lives of people all over the world and to know that, through science and medicine, we are truly making a difference. There are two parts to it – I call it the ‘what’ and the ‘how.’ ‘What’ we develop, the innovative products – whether in oncology, vaccines, or other areas – that make a real difference. And ‘how’ we act, how we make decisions in ways that keep patients at the center of our actions, is a relentless focus that continues to drive me.

You’re passionate about the importance of sharing clinical trial data. How do you think it can be done in an effective, ethical way?

I believe that sharing clinical trial data advances the science that is the foundation of medicine. It also honors the participants who devoted their time and effort by giving their data new life, to answer questions beyond the intent of the original clinical trial.

Sharing data has to be done in a responsible way, that protects privacy, and also assures that research proposals are reviewed fairly. Since 2014, clinical trial data from the Johnson & Johnson Family of
Companies have been shared with researchers around the world through the Yale University Open Data Access (YODA) Project. The YODA Project conducts a fair and unbiased assessment of outside research proposals that require the use of clinical trial data.

The YODA Project staff has full decision rights regarding granting access to clinical trial data from studies conducted by Johnson & Johnson. We also diligently protect patient privacy by de-identifying the data and allowing access through a secure server.

The landscape of health care and research is so exciting and innovative. How do we keep patient centricity and safety at the core?

Healthcare is undergoing unprecedented change, with advancements in biology, technologies, data analytics, and patients and caregivers that are increasingly educated and engaged in their own care. As healthcare professionals, I believe that we have a responsibility to engage directly with patients, not just work for patients – we should be seeking out input, listening to and incorporating patient perspectives into our work and decision-making. It’s imperative that we view patients and caregivers as partners and drivers.

At Johnson & Johnson, we recognized the opportunity to systematically change the way we work to meet the changing needs of patients and consumers. We embarked on a multi-year journey to establish a strong Patient Engagement model where we engage patients early, systematically and directly across important aspects of drug development and treatment – starting with R&D and pre-approval patient access programs through to when a medicine is approved and available by prescription.

Through this model, we work to integrate patient perspectives as early as possible to guide our decisions. For example, we were increasingly seeing critically ill patients and their loved ones self-advocating for access to medications before they were approved by the FDA (“pre-approval”). Johnson & Johnson saw a need to develop a new pathway to ensure pre-approval access decisions were approached through a consistent, transparent and equitable process.

Three years ago, we partnered with the Division of Medical Ethics at New York University (NYU) to create the Compassionate Use Advisory Committee (CompAC) model.

Through this effort, decisions about pre-approval access – which were previously made internally, without any external input – are now informed by an external, independent group of experts which include external medical experts, bioethicists and importantly, patient representatives. In this way we created a process where all patient and family requests are treated fairly, without regard to socioeconomic status, and patient representatives are at the table.

What are issues or topics that you’d like to see more conversation around and action going towards in the patient sphere?

I believe that this is the most exciting time to be working in science and medicine. We are on the verge of taking big steps in transformational approaches to treatment and prevention. Along with these advances, we need to include patients and caregivers in designing more impactful endpoints for products.

While many endpoints are important from a regulatory perspective, both healthcare companies and regulators need to work with people to really understand what is most meaningful for them in their journey – and strive to impact those endpoints.

Another topic that requires further conversation is understanding the health needs of diverse populations. Big data, in particular, can be extremely impactful in understanding the effectiveness and safety of different treatment pathways across more diverse populations.

Finally, I want to see continued conversation and prioritization of efforts around prevention. Many conditions can be more easily treated when they are caught early, or prevented entirely. I believe that this is a very important area of future dialogue and investment.

Joanne Waldstreicher, MD, is Chief Medical Officer, Johnson & Johnson, where she has oversight across pharmaceuticals, devices and consumer products for safety, epidemiology, clinical and regulatory operations transformation.

Among her prior roles, Dr Waldstreicher was responsible for late-stage development in neuroscience, cardiovascular disease and metabolism at Janssen. Before joining Johnson & Johnson in 2002, she headed endocrinology and metabolism clinical research at Merck Research Laboratories, overseeing development programs in atherosclerosis, obesity, diabetes, urology and dermatology.

Dr Waldstreicher graduated cum laude from Harvard Medical School, completed her internship and residency at Beth Israel Hospital and endocrinology fellowship at Massachusetts General Hospital.
You worked for a clinical trial recruitment company when you were diagnosed with Stage IV melanoma; how do you think that contributed to your advocacy?

The entire reason I became an advocate was because I had all this knowledge about clinical trials. So when I was diagnosed, I didn’t need to spend time getting up to speed on what a clinical trial was. It was more, “Which trial should I join?” and not “Should I join a trial?”

Then as I was meeting other patients who were being told by their doctors, “I have nothing else for you,” “You have months to live,” “Get your affairs in order,” and who were desperately trying to research anything they could on their own, without any firsthand knowledge of clinical trials, how to access them or how to understand them, I was realizing that that’s an enormous gap for the regular person.

Unless their oncologist brought up clinical trials to them, which is very few and far between. Only like 10% of non-research oncologists talk about clinical trials to their patients. If that conversation doesn’t happen, then a patient’s not even going to know to look for clinical trials.

The whole reason I got into advocacy work was because, as I was blogging my experience, other patients were contacting me because they had been told by their oncologist that they’ve done chemotherapy, they’ve done it all, and there’s nothing else that can be done and “Get your affairs in order.”

Melanoma can skew younger and it’s often young mothers. So that’s not enough. People are desperately researching anything they can online, to find options. People would stumble across my blog and then contact me. I knew as an industry statistic that oncologists do not refer to clinical trials, but I didn’t personalize it, internalize that information until I got the onslaught of requests from other patients, who were desperate to find information on their own. Their doctors had told them there were no options for them without even mentioning clinical trials.

What was involved with your advocacy?

I blogged the whole trial in real-time to help raise awareness of clinical trials and information to patients because I wasn’t working. So I felt like I wanted to be doing something to help the industry while I was sick. Then patients started contacting me and they were desperately trying to figure out their own pathways and stumbling across my blog.

I was working with a lot of patients one-on-one to help them understand clinical trials, navigate treatment options. I started getting involved with some of the advocacy groups for melanoma. Through that, I started making other connections; I started working with some pharmaceutical companies, helping them to write information about clinical trials in ways that patients could understand.

My passion, my soapbox if you will, as an advocate is the need to get clinical trial information into the hands of patients, not using doctors as gatekeepers. That’s when I started speaking at conferences, to help physicians and pharmaceutical companies understand how the referral process really works.

Non-research oncologists do not refer to clinical trials, and only 5% of people with cancer participate in clinical research. My road to advocacy just started with helping patients. The more I realized how few referrals were really happening, and what kinds of conversations were happening between medical oncologists and their patients, and that they did not include clinical trial information as much as they should, I became more and more passionate about the need to spread information.

How would you describe a clinical trial to someone who has never heard of one?

I would say a clinical trial is the process that drugs and medication go through before they’re available on the market for the general public. They’re mandated by the FDA to test whether an investigational treatment is safe and effective and...
Patients as Partners Celebrates

Jamie Troil Goldfarb is a metastatic melanoma cancer survivor, mother, blogger and clinical trials advocate. She was diagnosed with Stage IV melanoma in 2011, after previously being diagnosed with Stage II more than 3 years earlier after a changing birthmark was diagnosed as a tumor.

Is there anything you’d like to end with?

I would stress the fact that the only way patients can join clinical trials is if they know about them, and they only know about them if information about them is available where they’re searching in ways that they can understand. We, patients, desperately need more emphasis on clinical trials as viable treatment options, especially for life-threatening illnesses like cancer.

Be bold and confident in the fact that clinical trials are viable treatment options, and present them as thus to your patients.

What would you recommend for someone looking to make an impact with advocacy?

I would start with the advocacy groups for the condition that they’re passionate about. Those advocacy groups have patient forums where you can interact with other patients, you can help answer questions, you can spread your own story to raise awareness. And then the more you get involved with advocacy groups, the more opportunities there are to make your voice heard because advocacy groups are often contacted by journalists and other kinds of media who need to tell stories and need patient voices included.

There’s conferences that advocacy groups have that combine patients and physicians to help share information together. It’s really just about starting there and then making all the connections that you can and making sure that you’re where your cause is.

Is there anything pharma could be doing?

Pharma spends millions of dollars in patient recruitment for every trial. If they could pull some of those resources and do a general public awareness campaign about clinical trials, that would have an enormous impact on recruitment. The general public doesn’t understand clinical trials; they see it as being a guinea pig or that your hair’s going to turn green.

People say things like, “Why would I do an investigational treatment when I have a proven treatment available today?” Well, an investigational treatment is just an approved treatment that hasn’t been approved yet. Investigational treatment means that the research is moving forward and that you are getting access to the most cutting-edge treatments available, just in an investigational setting.

If the drugs that already exist were good enough, they wouldn’t need investigational treatments.

should be approved for general distribution. Every single drug and medication on the market has gone through clinical trials.

Pharma spends millions of dollars in patient recruitment for every trial. If they could pull some of those resources and do a general public awareness campaign about clinical trials, that would have an enormous impact on recruitment. The general public doesn’t understand clinical trials; they see it as being a guinea pig or that your hair’s going to turn green.

People say things like, “Why would I do an investigational treatment when I have a proven treatment available today?” Well, an investigational treatment is just an approved treatment that hasn’t been approved yet. Investigational treatment means that the research is moving forward and that you are getting access to the most cutting-edge treatments available, just in an investigational setting.

If the drugs that already exist were good enough, they wouldn’t need investigational treatments.

Is there anything pharma could be doing?

Pharma spends millions of dollars in patient recruitment for every trial. If they could pull some of those resources and do a general public awareness campaign about clinical trials, that would have an enormous impact on recruitment. The general public doesn’t understand clinical trials; they see it as being a guinea pig or that your hair’s going to turn green.

People say things like, “Why would I do an investigational treatment when I have a proven treatment available today?” Well, an investigational treatment is just an approved treatment that hasn’t been approved yet. Investigational treatment means that the research is moving forward and that you are getting access to the most cutting-edge treatments available, just in an investigational setting.

If the drugs that already exist were good enough, they wouldn’t need investigational treatments.
Lesley Gosden
Patient Advocate and Parkinson’s Patient

Why did you get involved as a patient advocate?
I think the reason I got involved – I mean, before I went on the trial, I’d done various small trials, one I answered questionnaires and did tap tests on computers, had electrodes on my head. Nothing that was as invasive as this one. And I think because we became very involved with the clinical team that were running it and very involved with the other patients, I think you almost feel that you’re very much a part of the trial. It was like a little family, all of us, and we looked out for each other. You very much wanted it to work, because of the trial team and the work that’s gone into it.

So then when you come out of the trial at the other end, and you know that the treatment works, but because of the trial design it hasn’t been proven, it’s the sheer frustration of knowing that there is something out there that can stop the condition. But it hasn’t been proved by science.

If only patients had had an input to that initial trial design, there are so many things that we would have changed, that could have made a difference. It was another thing that made me jump up and down so much, just sheer frustration that we see these conferences and speeches by the scientists saying, “Well, at the moment, there’s no definitive way to stop the progression of Parkinson’s or reverse the symptoms.” And we know that actually is, we just need to do some more work on it.

So I think that’s why I’ve gotten so involved in it. And because the group has stayed together afterwards, more for moral support than anything else, you live and breathe it all the time.

What do you as an advocate?
I go around local Parkinson’s UK branches; that’s where people with Parkinson’s meet up, generally once a week. They have speakers and they have presentations. So I go around the country talking to those groups; I’ve done quite a few of those already. I’ve spoken at research presentations, when there’s a regional meeting for Parkinson’s UK, again addressing patients.

I’m a PPI volunteer, which means I get involved in trial design. So if a researcher has an idea for a particular piece of research, they’ll come to Parkinson’s UK and ask for a PPI volunteer to work with. Then you look at the trial design, see whether the research is worthwhile, would it help people with Parkinson’s, whether it’s high priority. And then you look at the trial design itself, to see where there’s anything obvious in it that isn’t going to work for people with Parkinson’s.

I’m also a lay grant reviewer, which is looking at people that have applied to Parkinson’s UK for funding. And you look at the proposals and see, again, whether or not they’ll work for the patients, and then whether or not you think they’re worth funding.

With the GDNF group itself, we’re very much campaigning alongside Parkinson’s UK to get another trial running of GDNF, so we’re doing an awful lot of fundraising. I’ve done peer mentoring for people going into research projects, and I’m working on various projects, again, with Parkinson’s UK. One is on patient reported outcomes, and another one on a trial that should be starting up in the new year, hopefully.

I go along to Parkinson’s UK and I talk to their newly recruited staff about living with Parkinson’s and taking part in research. I’m also taking part in three different other research trials at the moment, fairly low-key compared to GDNF but research all the same.

How would you encourage other Parkinson’s advocates to get involved?
Parkinson’s UK runs the research support network, which is a network of people with Parkinson’s. They send out details of all research that’s going on, and that’s how they generally recruit volunteers; the PPI volunteers and the lay grant volunteers all stem from
Patients as Partners Celebrates

Lesley Gosden is a Parkinson’s patient and advocate. Lesley was diagnosed with Parkinson’s in 2004 when she was 43, and took ill health retirement in 2015. She participated in the GDNF trial. After the trial’s failure, Ms Gosden has remained a passionate advocate.

Her advocacy has included presenting at talks about the trial, working as a lay grant reviewer and working with a pharmaceutical to review assessment tools for Parkinson’s.

New Patients as Partners US dates:
June 29-30, 2020
Hilton Philadelphia at Penn’s Landing, Philadelphia, PA

Produced by Danny McCarthy

the research network.

They also have what they call the patient hub on the website, which is where they have a database of all the various trials that are going on around the country, whether they’re recruiting, who they’re looking for, what the eligible criteria is. There’s plenty of ways that you can get involved. Or talking to your local branch, because they’re fairly clued up on what’s going on in the community.

Have you noticed a change in the way people are designing trials?

No, unfortunately, it hasn’t. It’s not for the want of trying. There’s various different projects, but the wheels of research, and trying to change the regulatory bodies, grind very, very slowly. There’s lots in the pipeline, but I don’t think it’s actually hit at the root source of where the research ideas are coming from, and where the trial design is.

I think it’s slowly seeping through. There’s various researchers that certainly I’ve spoken to, who’ve said how they have gained so much from talking to patients and things they hadn’t thought about before. When you tell them, they’re completely obvious but at the time the researchers don’t think of it.

We need to make people that funding research, get in place procedures to make sure that patients are involved from the outset. And that is slow progress at the moment.

What do you think makes for a more patient-centric trial?

I think it’s very simple. At the end of the day, all you want to do is improve quality of life, and however that’s achieved. You don’t really need to look into the symptoms and pinpoint exactly what’s changed and what hasn’t changed. I think overall, if quality of life has improved, that it’s a success.

I know clinical trials have to go into all the nitty-gritty details and mark off which symptoms are better and which symptoms are worse, but from a patient point of view, it’s an overall quality of life. If that’s improved, then all the better.

Is there anything else you’d like to include?

Patients need to be part of the trial process right from the beginning; it’s no good getting them involved once the design is up and running.

The mistakes that were made from the GDNF trial: one of the major problems we’ve got now is that there was no provision made for aftercare. It is so important; you can’t just end a trial and leave forty-odd people with a head full of equipment. Bearing in mind, it was completely novel equipment that’s never been tried before. So you go to your local hospital and say, “I’ve got a headache,” and obviously they’re not going to know, because they don’t know what you’ve got in there.

I think they need to think ahead to when the trial finishes, as well. I think they’re inclined to finish the trial and think, “Well, that’s it. Off you go. The trial’s finished,” but for the patient, it doesn’t end there. That’s one of the things we’re campaigning quite heavily on. There needs to be provisions for patients to be looked after at the end of the trial as well, not just during the trial.